

107TH CONGRESS
1ST SESSION

S. 1379

To amend the Public Health Service Act to establish an Office of Rare Diseases at the National Institutes of Health, and for other purposes.

IN THE SENATE OF THE UNITED STATES

AUGUST 3, 2001

Mr. KENNEDY (for himself and Mr. HATCH) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To amend the Public Health Service Act to establish an Office of Rare Diseases at the National Institutes of Health, and for other purposes.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Rare Diseases Act of
5 2001”.

6 **SEC. 2. FINDINGS AND PURPOSES.**

7 (a) FINDINGS.—Congress makes the following find-
8 ings:

1 (1) Rare diseases and disorders are those which
2 affect small patient populations, typically popu-
3 lations smaller than 200,000 individuals in the
4 United States. Such diseases and conditions include
5 Huntington’s disease, amyotrophic lateral sclerosis
6 (Lou Gehrig’s disease), Tourette syndrome, Crohn’s
7 disease, cystic fibrosis, cystinosis, and Duchenne
8 muscular dystrophy.

9 (2) For many years, the 25,000,000 Americans
10 suffering from the over 6,000 rare diseases and dis-
11 orders were denied access to effective medicines be-
12 cause prescription drug manufacturers could rarely
13 make a profit from marketing drugs for such small
14 groups of patients. The prescription drug industry
15 did not adequately fund research into such treat-
16 ments. Despite the urgent health need for these
17 medicines, they came to be known as “orphan
18 drugs” because no companies would commercialize
19 them.

20 (3) During the 1970s, an organization called
21 the National Organization for Rare Disorders
22 (NORD) was founded to provide services and to
23 lobby on behalf of patients with rare diseases and
24 disorders. NORD was instrumental in pressing Con-

1 gress for legislation to encourage the development of
2 orphan drugs.

3 (4) The Orphan Drug Act created financial in-
4 centives for the research and production of such or-
5 phan drugs. New federal programs at the National
6 Institutes of Health and the Food and Drug Admin-
7 istration encouraged clinical research and commer-
8 cial product development for products that target
9 rare diseases. An Orphan Products Board was estab-
10 lished to promote the development of drugs and de-
11 vices for rare diseases or disorders.

12 (5) Before 1983, some 38 orphan drugs had
13 been developed. Since the enactment of the Orphan
14 Drug Act, more than 220 new orphan drugs have
15 been approved and marketed in the United States
16 and more than 800 additional drugs are in the re-
17 search pipeline.

18 (6) Despite the tremendous success of the Or-
19 phan Drug Act, rare diseases and disorders deserve
20 greater emphasis in the national biomedical research
21 enterprise. The Office of Rare Diseases at the Na-
22 tional Institutes of Health was created in 1993, but
23 lacks a statutory authorization.

24 (7) The National Institutes of Health has re-
25 ceived a substantial increase in research funding

1 from Congress for the purpose of expanding the na-
2 tional investment of the United States in behavioral
3 and biomedical research.

4 (8) Notwithstanding such increases, funding for
5 rare diseases and disorders at the National Insti-
6 tutes of Health has not increased appreciably.

7 (9) To redress this oversight, the Department
8 of Health and Human Services has proposed the es-
9 tablishment of a network of regional centers of excel-
10 lence for research on rare diseases.

11 (10) The Food and Drug Administration sup-
12 ports small clinical trials through Orphan Products
13 Research Grants. Such grants embody successful
14 partnerships of government and industry, and have
15 led to the development of at least 23 drugs and four
16 medical devices for rare diseases and disorders. Yet
17 the appropriations in Fiscal Year 2001 for such
18 grants were less than in Fiscal Year 1995.

19 (b) PURPOSES.—The purposes of this Act are to—

20 (1) amend the Public Health Service Act to es-
21 tablish an Office of Rare Diseases at the National
22 Institutes of Health; and

23 (2) increase the national investment in the de-
24 velopment of diagnostics and treatments for patients
25 with rare diseases and disorders.

1 **TITLE I—NATIONAL INSTITUTES**
2 **OF HEALTH**

3 **SEC. 101. NIH OFFICE OF RARE DISEASES.**

4 Title IV of the Public Health Service Act (42 U.S.C.
5 281 et seq.) is amended by inserting after section 404D
6 the following:

7 “OFFICE OF RARE DISEASES

8 “SEC. 404E. (a) ESTABLISHMENT.—There is estab-
9 lished within the Office of the Director of NIH an office
10 to be known as the Office of Rare Diseases (in this section
11 referred to as the ‘Office’), which shall be headed by a
12 Director (in this section referred to as the ‘Director’), ap-
13 pointed by the Director of NIH.

14 “(b) DUTIES.—

15 “(1) IN GENERAL.—The Director of the Office
16 shall carry out the following:

17 “(A) The Director shall recommend an
18 agenda for conducting and supporting research
19 on rare diseases through the national research
20 institutes and centers. The agenda shall provide
21 for a broad range of research and education ac-
22 tivities, including scientific workshops and
23 symposia to identify research opportunities for
24 rare diseases.

1 “(B) The Director shall, with respect to
2 rare diseases, promote coordination and co-
3 operation among the national research insti-
4 tutes and centers and entities whose research is
5 supported by such institutes.

6 “(C) The Director shall enter into coopera-
7 tive agreements with and make grants for re-
8 gional centers of excellence on rare diseases in
9 accordance with section 404F.

10 “(D) The Director shall promote the suffi-
11 cient allocation of the resources of the National
12 Institutes of Health for conducting and sup-
13 porting research on rare diseases.

14 “(E) The Director shall promote and en-
15 courage the establishment of a centralized
16 clearinghouse for rare and genetic disease infor-
17 mation that will provide understandable infor-
18 mation about these diseases to the public, med-
19 ical professionals, patients and families.

20 “(F) The Director shall biennially prepare
21 a report that describes the research and edu-
22 cation activities on rare diseases being con-
23 ducted or supported through the national re-
24 search institutes and centers, and that identi-
25 fies particular projects or types of projects that

1 should in the future be conducted or supported
2 by the national research institutes and centers
3 or other entities in the field of research on rare
4 diseases.

5 “(G) The Director shall prepare the NIH
6 Director’s annual report to Congress on rare
7 disease research conducted by or supported
8 through the national research institutes and
9 centers.

10 “(2) PRINCIPAL ADVISOR REGARDING ORPHAN
11 DISEASES.—With respect to rare diseases, the Direc-
12 tor shall serve as the principal advisor to the Direc-
13 tor of NIH and shall provide advice to other relevant
14 agencies. The Director shall provide liaison with na-
15 tional and international patient, health and scientific
16 organizations concerned with rare diseases.

17 “(c) DEFINITION.—For purposes of this section, the
18 term ‘rare disease’ means any disease or condition that
19 affects less than 200,000 persons in the United States.

20 “(d) AUTHORIZATION OF APPROPRIATIONS.—For the
21 purpose of carrying out this section, there are authorized
22 to be appropriated \$4,000,000 for fiscal year 2002, and
23 such sums as may be necessary for each subsequent fiscal
24 year.”.

1 **SEC. 102. RARE DISEASE REGIONAL CENTERS OF EXCEL-**
 2 **LENCE.**

3 Title IV of the Public Health Service Act (42 U.S.C.
 4 281 et seq.), as amended by section 101, is further amend-
 5 ed by inserting after section 404E the following:

6 “RARE DISEASE REGIONAL CENTERS OF EXCELLENCE

7 “SEC. 404F. (a) COOPERATIVE AGREEMENTS AND
 8 GRANTS.—

9 “(1) IN GENERAL.—The Director of the Office
 10 of Rare Diseases (in this section referred to as the
 11 ‘Director’) shall enter into cooperative agreements
 12 with and make grants to public or private nonprofit
 13 entities to pay all or part of the cost of planning, es-
 14 tablishing, or strengthening, and providing basic op-
 15 erating support for regional centers of excellence for
 16 clinical research into, training in, and demonstration
 17 of diagnostic, prevention, control, and treatment
 18 methods for rare diseases.

19 “(2) POLICIES.—A cooperative agreement or
 20 grant under paragraph (1) shall be entered into in
 21 accordance with policies established by the Director
 22 of NIH.

23 “(b) COORDINATION WITH OTHER INSTITUTES.—
 24 The Director shall coordinate the activities under this sec-
 25 tion with similar activities conducted by other national re-
 26 search institutes, centers and agencies of the National In-

stitutes of Health and by the Food and Drug Administration to the extent that such institutes, centers and agencies have responsibilities that are related to rare diseases.

“(c) USES FOR FEDERAL PAYMENTS UNDER COOPERATIVE AGREEMENTS OR GRANTS.—Federal payments made under a cooperative agreement or grant under subsection (a) may be used for—

“(1) staffing, administrative, and other basic operating costs, including such patient care costs as are required for research;

“(2) clinical training, including training for allied health professionals, continuing education for health professionals and allied health professions personnel, and information programs for the public with respect to rare diseases; and

“(3) clinical research and demonstration programs.

“(d) PERIOD OF SUPPORT; ADDITIONAL PERIODS.—Support of a center under subsection (a) may be for a period of not to exceed 5 years. Such period may be extended by the Director for additional periods of not more than 5 years if the operations of such center have been reviewed by an appropriate technical and scientific peer review group established by the Director and if such group

1 has recommended to the Director that such period should
2 be extended.

3 “(e) AUTHORIZATION OF APPROPRIATIONS.—For the
4 purpose of carrying out this section, there are authorized
5 to be appropriated \$20,000,000 for fiscal year 2002, and
6 such sums as may be necessary for each subsequent fiscal
7 year.”.

8 **TITLE II—FOOD AND DRUG** 9 **ADMINISTRATION**

10 **SEC. 201. GRANTS AND CONTRACTS FOR THE DEVELOP-** 11 **MENT OF ORPHAN DRUGS.**

12 Subsection (c) of section 5 of the Orphan Drug Act
13 (21 U.S.C. 360ee(c)) is amended to read as follows:

14 “(c) For grants and contracts under subsection (a)
15 there are authorized to be appropriated \$25,000,000 for
16 fiscal year 2002, and such sums as may be necessary for
17 each subsequent fiscal year.”.

18 **SEC. 202. TECHNICAL AMENDMENT.**

19 Section 527(a) of the Federal Food, Drug, and Cos-
20 metic Act (21 U.S.C.360cc(a)) is amended in the matter
21 following paragraph (2)—

22 (1) by striking “, of such certification,”; and

23 (2) by striking “, the issuance of the certifi-
24 cation,”.

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